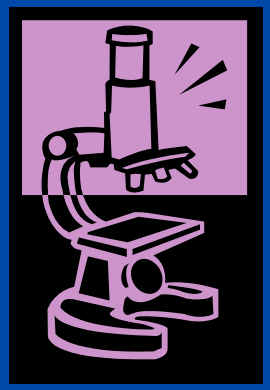
New Models in Collaborative R & D



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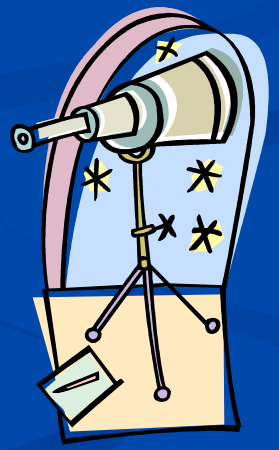


NASA's Turning Goals into Reality, June 10-12, 2003

27 Institutes & Centers at NIH

Budget Allocations

NIH Budget FY 2002

Total Budget:	23.5 billion
Extramural:	21.3 billion
Intramural	2.2 billion

NCI Budget FY2002

Total budget	4.11 billion
Extramural	3.47 billion
Intramural	635 million

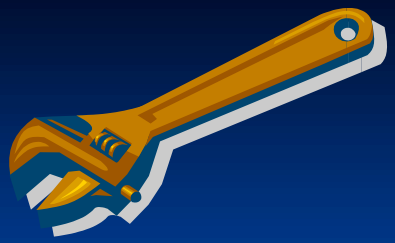
10-15 % of the budget is used for Intramural Research.

NCI Mission

" Conducts and supports research, training, health information dissemination, and other programs with respect to the cause, diagnosis, prevention, and treatment of cancer, rehabilitation from cancer, and the continuing care of cancer patients and the families of cancer patients."

The Molecular Revolution & Cancer Research

- Unveiling the molecular differences
  - **Not one disease**
- Discovering associated genes and their mutations
- Deciphering of the complex networks of cellular communications; proteins and pathways
- Drug design both practical and virtual



Tools of this Trade

- Molecular Biology (Genetics)
- Protein Chemistry and Biology (Proteomics)
- General Chemistry (drug synthesis, high throughput screening, and development)
- Methods of drug delivery
- Clinical Resources
- Bioinformatics

First Focus: Intramural Collaboration

Key issues for NCI

- Partnering to:
  - Bring technology in for NCI use
  - Move NCI developed technology out for public use
- Publication and dissemination
- Appropriate IP management
- Return on taxpayer investment

Cooperative Research and Development Agreements (CRADAs)

- Basic Research CRADA
- Specialized Materials CRADA
- Clinical Trial CRADA

CRADA

- A focused collaboration, typically with for-profit
- A CRADA provides the Collaborator with an option to an exclusive license to CRADA subject inventions
- A CRADA is the only mechanism the NIH currently has to promise NIH intellectual property rights in advance

CRADA

- The Federal laboratory may provide
  - Personnel
  - Services
  - Facilities, equipment, or other resources
- The Collaborator may provide
  - Funds
  - Personnel
  - Services
  - Facilities, equipment, or other resources

Materials CRADA

- Receipt of proprietary Materials for Research Purposes
- No other scientific interaction with the outside party
- Not for Human use
- Promise license option to future inventions by NIH Scientist
- One year duration
- Minimal funding from outside party (Not to exceed $20,000)
- Shorter agreement and approval process

Clinical Trial CRADA

- Collaborator wants Rights to Future Inventions by NIH Scientist
- Collaboration involves Clinical Trials; early or late phase
  - Collaborator requires Exclusive Access to Drug, Technology, Data, Expertise from NCI
  - Addresses IND
  - Takes advantage of NCI extensive network of clinical centers
- Significant funding may be received by NIH

Second Focus: Extramural Funding to Foster Collaborations

Key issues for NCI

- Development of platform technologies to support the diagnostic/therapeutic needs of the patients
- Provide resources to accelerate the clinical testing needed to bring into commercialization
- Share Research Tools to accelerate the rate of developing new diagnostics and therapeutics
- Communication of results in a timely manner to the public
- Support of underserved cancer types

Mouse Model of Human Cancer Consortium: GOALS

- To Derive and characterize mouse models of human cancer and to generate resources, information and innovative approaches to the application of mouse models in cancer research.
- By correlating animal model data and human data, the results of this consortium could impact how companies conduct basic, preclinical and clinical development in the future such as:

develop preventive strategies

develop improved imaging strategies

develop new early detection systems

discover and test new therapies

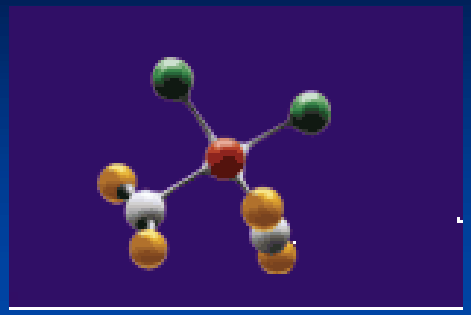
MMHCC

Mouse Models of Human Cancers Consortium

MMHCC Set-up

- Consortium of 19 Cooperative Agreement grantees from non-profits and companies and 1 intramural NCI member
- Investigators are funded for 1 year renewable up to 5 years, ($500,000 in direct costs)
- Each of the 20 groups has its own collaborators
- Connects over 40 institutions in the U.S. and abroad
- No co-funding from other institutions
- Grantee Institutions retain their IP rights under Bayh-Dole Act (35 U.S.C. § 202)
- Public sharing of the data through web-based database
- NCI contractor maintains the repository of the mice for distribution
- Specialized agreements for distribution of the mice

Initiative in Chemical Genetics (ICG)



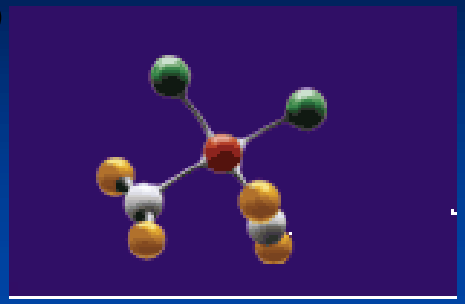
Goals:

The ICG [formerly Molecular Targets Laboratory (MTL)] focus is to develop a resource of biological assays and chemical probes for the study of cancer.

The ICG will:

- emphasize the need for collaboration between government, industry and academia in the fields of chemistry, biology and bioinformatics in an effort to produce **libraries** of potential anti-cancer compounds for public distribution
- develop **screening assays** suitable for high-throughput screening of chemical libraries of potential agents
- Provide **confirmation** a drug's initial ability to alter the drug target in cancer cells.
- Make data available to public as quickly as possible

ICG Parameters



- Contract
- First round awarded to Harvard
- Provides guidelines for sharing all data
- NCI Sponsored IP Committee
- An approved Determination of Exceptional Circumstances (DEC) to the Bayh-Dole Act, but uniquely managed.
  - Recognizes and provides guidelines for possibility of patent protection for some of the technologies developed

Rapid Access to Intervention Development (RAID)

The Rapid Access to Intervention Development

(RAID) program is designed to assist translation to

the clinic of novel anticancer therapeutic

interventions, either synthetic, natural product, or

biologic, arising in the academic community.

RAID Parameters

- No funding
- Provides NCI resources including our contract resources:
  - Small- medium scale production
  - Bulk supply
  - GMP manufacturing
  - formulation
  - Toxicology
- IP: GOCO and any subcontract it uses for the project are DEC'd. Most IP developed flows to Government to allow it to be unencumbered by such third party contracts for use by the RAID Recipient.

Academic Public Private Partnership Program (AP4)

- To stimulate cancer intervention discovery and development research at academic centers in partnership with industry, non-profits and government
- Focus on:
  - Incorporation of the latest technologies and
  - novel, mechanistically targeted drugs and other intervention strategies for underserved diseases
- To bring together the necessary expertise to reduce the time required to translate novel drug discoveries into therapies.

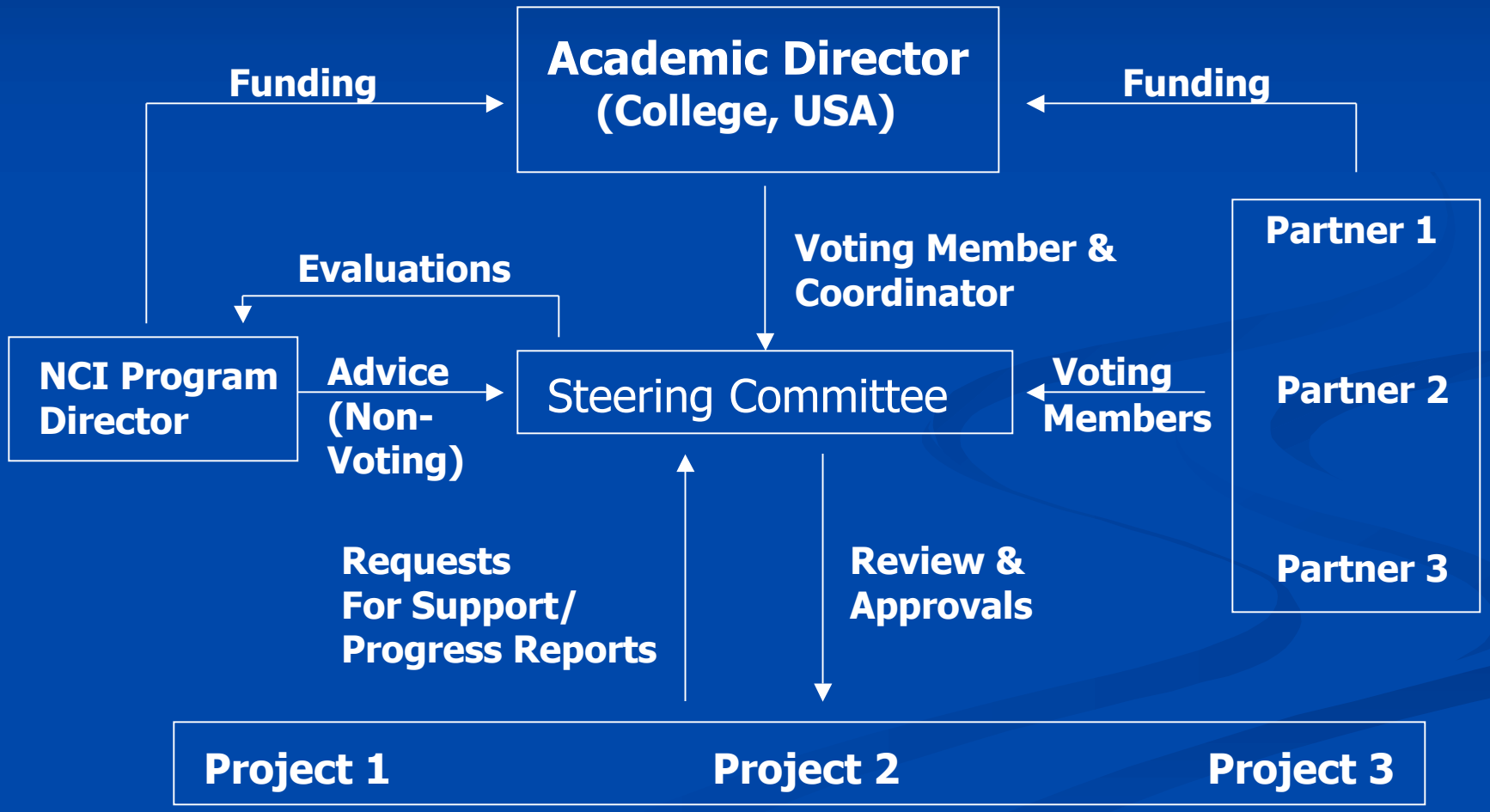
AP4 Predecessor

- Modeled after Industrial/University Cooperative Research Centers (I/UCRC) at National Science Foundation which stimulates industrial-academic partnerships
- IP is managed based on the membership agreement generated by the Academic Director and agreed to upfront by the “partners”
- Initially Funded through 5 year Cooperative Agreements from NCI. Designed such that by year 5, the NCI funding of the Center will have been decreased to 50 %. It is expected that the Centers’ partners will take over the difference + in funding. The goal is for the Center to become essentially self-sufficient by the end of 5 years.

AP4 Parameters

- Academic Director
- Each Center can have many “partners” - other academics, small or large pharma, biotechs, non-profits
- Academic Director generates a membership agreement – governance document
- Steering Committee - chaired by Academic Director; actually governs the Center – represents partners + NCI
- Focus is preclinical discovery and early development but with facile access to NCI late development and clinical resources, and possible support for ancillary related studies.
- Dynamic project management

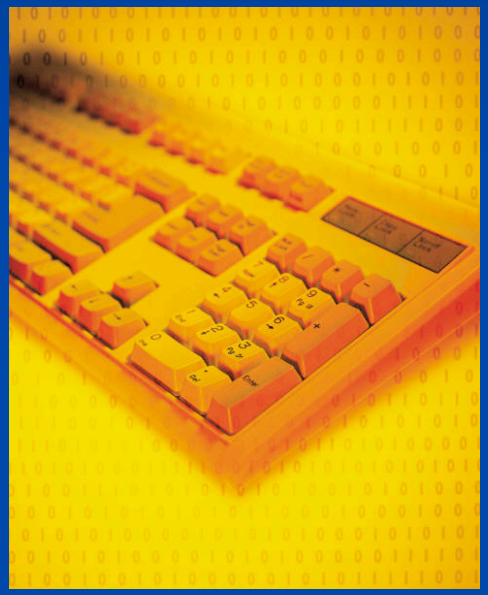
AP4 Interactive Flow



AP4 Summary

The AP4 concept is unique to NCI– provides a focus on partnering, new technologies, and underserved diseases, with Gov't and members sharing costs; Steering Committee vested with the power to make immediate go/no go decisions.

Useful Websites



- For RAID and AP4: http://dtp.nci.nih.gov/index.html
- For ICG: http://iccb.med.harvard.edu/
- For MMHCC: http://mouse.ncifcrf.gov
- For Technology Transfer:
  NIH: http://ott.od.nih.gov
  NCI: http://ttb.nci.nih.gov